CRISPR/dCas9 mutant targeting SNCA promoter for downregulation of alpha-synuclein expression as a novel therapeutic approach for Parkinson's disease

Grant Award Details

CRISPR/dCas9 mutant targeting SNCA promoter for downregulation of alpha-synuclein expression as a novel therapeutic approach for Parkinson's disease

Grant Type: Quest - Discovery Stage Research Projects

Grant Number: DISC2-09610

Project Objective: CRISPR/dCas9 mutant targeting SNCA (alpha-synuclein) promoter for downregulation of alpha-synuclein expression as a novel therapeutic approach for Parkinson's disease.

Investigator:

Name:	Birgitt Schuele
Institution:	Parkinson's Institute
Type:	PI

Disease Focus: Neurological Disorders, Parkinson's Disease

Human Stem Cell Use: iPS Cell

Award Value: $1,931,495

Status: Active

Grant Application Details

Application Title: CRISPR/dCas9 mutant targeting SNCA promoter for downregulation of alpha-synuclein expression as a novel therapeutic approach for Parkinson's disease

Public Abstract:

Research Objective

Discovery of a novel therapeutic candidate for Parkinson's disease which modifies gene expression using human stem cell-derived neurons to halt the neurodegenerative disease process.

Impact

Stopping the neurodegenerative process of Parkinson's disease is a critical unmet medical need. Our approach is based on novel gene engineering technology that modifies expression of key target genes.

Major Proposed Activities

- Identification and engineering of therapeutic candidates that downregulate expression of test gene in human stem cell-derived neuronal precursor cells.
- Measurement of target gene downregulation in human stem cell-derived neuronal precursor cells and neurons with assessment of phenotype rescue.
- Testing downregulation of target gene using relevant pre-clinical model containing endogenous gene regulatory regions.
- Development of a Target Product Profile for advancement of the therapeutic candidate for CIRM partnering opportunity: translational research projects (TRAN).
- Preparation for stage appropriate regulatory meetings for subsequent CIRM pre-clinical application. Develop regulatory strategy with CIRM Clinical Advisory Panel.

Statement of Benefit to California:

Estimated 36,000-60,000 people in the State of California are affected with Parkinson's disease which is a neurodegenerative disease that causes a high degree of disability and financial burden for our health care system. This collaborative project will provide substantial benefits and values to the state of California and its citizens by developing new therapeutic candidates for the treatment of Parkinson's disease enabled by stem cell technologies and gene therapy.
